Reissuance of RFA-CA-17-031

- Limited Competition RFA: To renew the Center for International Blood and Marrow Transplant Research (CIBMTR) Registry

A Data Resource for Analyzing and Supporting Blood and Marrow Transplants and Cellular Immunotherapy Research

NIH NATIONAL CANCER INSTITUTE

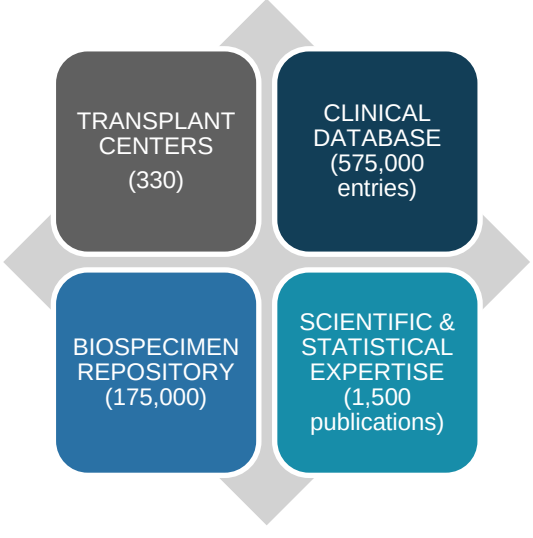
Lori Henderson, PhD and Rich Little, MD

Joint BSA/NCAB Meeting

December 9, 2021

A World-leading Resource that must remain available to the public

Funded by NCI for 30 plus years with support by NHLBI & NIAID



CIBMTR INTEGRATED NETWORK
(Data from 2020 ANNUAL REPORT)

CIBMTR is:

- The only publicly available, comprehensive U.S. Data Resource for Hematopoietic Stem Cell Transplant (HCT) & Adoptive Cell Therapy (ACT) research
- A unique national resource for researchers, clinicians, HHS policymakers, and pharmaceutical companies
- Needed to support FDA CAR T-cell product safety and efficacy data & CMS coverage with evidence development trials
- Uniquely suited to conduct observational studies that inform trial designs and clinical practice
- A network of multi-disciplinary, world leaders that has evolved to meet current medical research needs that can't be replicated elsewhere

CIBMTR Accomplishments (2018-2020)

RFA Benchmarks on Resource Development & Resource Utilization

- Enrolled ~72,000 new cell therapy patients
- Enrolled 5,000 recipients receiving Adoptive Cell Therapy (ACT) for transplants or as a primary therapy
- Adapted forms to collect COVID-19 data which produced 5 papers in leading journals
- Created new forms to capture data on solid tumors
- Published 267 data analysis and research manuscripts
- Distributed 13,000 samples for secondary research
- Enhanced data quality by training 85 data managers
- Piloted new technology platforms to automate data acquisition and reduce the burden of data entry
- Engaged in 5 Medicare Coverage with Evidence Development Trials

CAR T-cell Data Collection via Public-Private-Partnerships

A. Industry Partners - FDA required Post-Market Safety Reporting

- Launched 5 long-term follow-up studies since 2018

B. NCI-funded Collaborators - Linking clinical outcome data with proteomics and genomics research

- AIDS Malignancy Consortium (AMC)
- Blood and Marrow Clinical Trials Network (BMT CTN)
- National Clinical Trials Network (NCTN)

Model Program Linking Data Sets to Inform Clinical Practice

(1) Novartis Post-Market Studies confirms Eliana/Juliet Trial Results

Kymriah (Tisagenlecleucel) in Pediatric Acute Lymphoblastic Leukemia and Non-Hodgkin Lymphoma

- Toxicity is tolerable when administered with anti-inflammatory/anti-IL-6 therapeutics for serious immune toxicities
- Overall survival consistent with pivotal clinical trials

Pasquini, M., et. al., Blood Advances (2020)

(2) Multi-Omics integration project* examines Prognostic Determinants in Myelodysplastic Syndrome (MDS)

Data integration of 1,500 healthy donor-MDS recipient pairs – clinical, whole genome sequencing, epigenetic, and proteomic analyses

Initial Findings**:

- Shorter survival data associated with TP53, RAS, and JAK2 pathway mutations
- Adverse effects of RAS mutations were reduced by high intensity conditioning
- Proteomic analysis of chemokines may also predict disease response

**Department of Defense Grant*

***Lindsley, R.C., et.al., New England Journal of Medicine (2017); Saber Wael, et. al. Leukemia & Lymphoma (2021)*

Plans for the RFA Reissuance

- Expand HCT and ACT data collection used to treat malignant and non-malignant blood disorders
- Focus on special initiatives including HHS evidence gathering programs:
  - FDA required long-term follow-up studies with industry – Expected to be over 9000 ACT annually in next few years
  - CMS Medicare Coverage with Evidence Development Trials
- Adapt the database for the collection of ACT for solid tumors as well as new ACT products (as appropriate)
- Support trial designs and data analyses for observational and interventional studies

Thank you for your attention

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